

Title (en)
GENERATION OF IMPROVED HUMAN PAH FOR TREATMENT OF SEVERE PKU BY LIVER-DIRECTED GENE REPLACEMENT THERAPY

Title (de)
ERZEUGUNG EINER VERBESSERTEN MENSCHLICHEN PAH ZUR BEHANDLUNG VON SCHWERER PKU DURCH AUF DIE LEBER
GERICHTETE GENSATZTHERAPIE

Title (fr)
GÉNÉRATION DE PAH HUMAIN AMÉLIORÉ POUR LE TRAITEMENT D'UNE PKU SÉVÈRE AU MOYEN D'UNE THÉRAPIE DE
REMPLACEMENT GÉNIQUE DIRIGÉE DANS LE FOIE

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Application
EP 19797895 A 20191011

Priority
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Abstract (en)
[origin: WO2020077250A1] Provided herein are variant phenylalanine hydroxylase (PAH) polypeptides which are more stable and have greater activity than wild-type human PAH. Also provided are methods to treat phenylketonuria (PKU) and/or to reduce levels of phenylalanine in an individual in need thereof. Further provided herein are expression cassettes, vectors (e.g., rAAV vectors), viral particles, pharmaceutical compositions and kits for expressing the variant PAH polypeptide in an individual in need thereof.

IPC 8 full level
A61K 38/44 (2006.01); **C12N 15/864** (2006.01)

CPC (source: EP KR US)
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C12N 15/8645 (2013.01 - EP); **C12Y 114/16001** (2013.01 - EP KR US); **A61K 48/00** (2013.01 - US); **C12N 2750/14143** (2013.01 - KR)

Citation (search report)
See references of WO 2020077250A1

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WO 2020077250 A1 20200416; AR 116700 A1 20210602; AU 2019357039 A1 20210603; BR 112021006796 A2 20210720;
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JP 2022504740 A 20220113; KR 20210076051 A 20210623; MX 2021004213 A 20210716; PH 12021550794 A1 20211213;
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JP 2021519879 A 20191011; KR 20217013976 A 20191011; MX 2021004213 A 20191011; PH 12021550794 A 20210411;
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